

## Children's Hospital Foundation

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### NEWS RELEASE

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### **NEW ERA FOR OKLAHOMA CHILDREN LIVING WITH CYSTIC FIBROSIS**

*Rapidly Approaching a Cure for Cystic Fibrosis*

**OKLAHOMA CITY, OKLA. (February 3, 2012)** – Children in Oklahoma are responding positively to the first disease-modifying Cystic Fibrosis drug which will drastically change the lives of children living with this debilitating disease. This new drug corrects the basic defect causing Cystic Fibrosis for people with CF with a particular type of mutation. Currently, patients on the drug are significantly improving and it is possible that this therapy is on the brink of a cure for CF.

CF is a severe genetic disorder affecting the lungs and eventually other vital organs. Although most children are diagnosed by age 2, the disorder ultimately leads to an untimely death as a result of life-threatening lung infections. Roughly 4% of the CF patient population has CF due to the G551D mutation. With this specific mutation, a defective protein caused by CF does not function properly. Fortunately, the new drug successfully restores the function of the protein, which in turn improves lung function.

Dr. James Royall, M.D., CMRI C.R. Anthony Centennial Chair in Pulmonology and Director of the Oklahoma Cystic Fibrosis Center, understands the importance of vigilance and aggressive care for his young patients. He has ongoing research to collect data on patients with CF to better understand the illness and ultimately improve the care and survival of those with CF. The new drug development is a collaborative project including researchers across the country. The Oklahoma CF Center is a site within this network of CF Centers that cooperates in the study of new drugs and therapy for CF. The Oklahoma CF Center participated in some studies that led to the approval of this new drug. Dr. Royall has been involved with CF for over 30 years. He came to Oklahoma 15 years ago from the University of Iowa to further his research with endowed funds made possible through Children's Hospital Foundation, the C.R. Anthony family and matching funds from the State of Oklahoma program for endowments.

"Thirty years ago, children with CF rarely lived past childhood. I really didn't expect a cure in my lifetime until five years ago when we could see the first positive results from an organized research and development process of new drug discovery," said Dr. Royall.

The state matching funds program was crucial for creating the endowed chair for Dr. Royall.

"He simply would not be in Oklahoma, caring for kids with Cystic Fibrosis and other lung diseases, and participating in research programs without the state matching funds program," said Kathy McCracken, Executive Director of Children's Hospital Foundation.

Unfortunately, the program no longer exists and Children's Hospital Foundation is stretching donors to fill the gap in order to recruit more specialists for Oklahoma's children.

Although the breakthrough treatment is targeted for this specific mutation, it brings hope to all people with CF as it provides gateways to additional treatments. Dr. Royall has 160 CF patients in Oklahoma ranging from infants to 46 years old. With this new drug, cases of CF will decrease dramatically over the next few years.

"It has been a remarkable collaborative process that led to this breakthrough and it would not have happened without research funding," said Dr. Royall. "My personal experience with patients in our CF Center is that this drug is working very, very well."

Children's Hospital Foundation helps to fund pediatric research, education and clinical care, ultimately supporting The Children's Hospital in serving every county in Oklahoma with more than 134,000 annual patient visits. All funds raised through Children's Hospital Foundation stay in Oklahoma so children will have access to exceptional pediatric specialists without having to leave the state. Despite dedicated and steady progress, there are still obstacles in the quest to combat pediatric illness and disability.

To donate to Children's Hospital Foundation, go to [www.okchf.org](http://www.okchf.org) or call 405.271.6050.

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ABOUT US: Children's Hospital Foundation, proud affiliate of Children's Miracle Network Hospitals, is a nonprofit 501c (3) organization in Oklahoma striving to improve the health of children. Since its inception in 1983, Children's Hospital Foundation, through its volunteer board and vast community support, has funded pediatric research and education programs including collaborative projects with the OU Department of Pediatrics, The Children's Hospital at OU Medical Center, OU Children's Physicians and the University Hospitals Authority and Trust. For more information, contact Executive Director, Kathy McCracken at 405.650.1718 or visit our website: [www.okchf.org](http://www.okchf.org).